February 14, 2018

Karen Reynolds
Executive Director
Office of Pharmaceuticals Management Strategies
Strategic Policy Branch, Health Canada
10th Floor, Brooke Claxton Building
70 Colombine Driveway, Tunney's Pasture
Ottawa, Ontario K1A 0K9

Re: Response to PMPRB Regulatory Proposals

Dear Ms. Reynolds;

The ALS Society of Canada (ALS Canada) is pleased to respond to the Proposed Amendments to the *Patented Medicines Regulations* in the Canada Gazette Part I. Specific feedback to the Amendments is provided in the attached submission by the Health Charities Coalition of Canada (HCCC).

ALS Canada and our provincial partners are dedicated to supporting Canadians living with ALS and investing in research to create a future without ALS. Approximately 3,000 Canadians are living with this terminal disease, which has a life expectancy of 2 to 5 years beyond diagnosis. While there is only one approved therapy for ALS in Canada, we anticipate that the next several years will see more Phase 3 clinical trials leading to new therapies for the first time in more than 20 years. We therefore have a strong interest in the proposed amendments to the patented medicines regulations given the need for Canadians living with ALS to be able to equitably access new therapies that have never before been an option.

We wish to draw your attention to the following four key points concerning the consultation process, as well as the potential impacts and outcomes to patients, as a result of the proposed modernization of the *Regulations*.

1. **Guiding Principles**
   Access to Medicines is an important issue to the members of the Health Charities Coalition of Canada (HCCC) and to the Canadians that they serve. The modernization of the *Patented Medicines Regulations* is one piece of the broader Federal Government plan to improve access, affordability, and appropriate prescribing of pharmaceuticals in Canada. HCCC's members have identified four guiding principles on access to medicines which should inform the Federal Government's plan, including the modernization of the *Patented Medicines Regulations*:

   - **Patient Partnerships** - Amendments to the *Patented Medicines Regulations* are developed, monitored, and evaluated in partnership with patients to ensure that the right medicine gets to the right patient at the right time in a cost-effective manner.

- **Quality** - Canadians deserve high-quality therapies and services that are appropriate for patients' needs, respect an individual's choice, and are delivered in a manner that is timely, safe, and effective according to current evidence.

- **Equity** - All Canadians should have equitable access to a comprehensive range of evidence-based medications to help meet their health needs, regardless of who they are, the setting they are in or where they live.

- **Sustainability** - That the implementation of the *Regulations* are evidence-based, adequately resourced, cost-effective for individuals, and are a sustainable element of the healthcare system that is continuously reviewed, evaluated, and improved.

We believe that meaningful solutions to gaining better access to medicines for Canadians will derive from the application of these principles. ALS Canada applauds Health Canada and the PMPRB for their desire to modernize the twenty-year old *Regulations* with a view to ensuring that effective and relevant regulatory tools are in place while remaining responsive to the continued introduction of innovative therapies in Canada.

2. Finding the Right Balance

The mandate of the Patented Medicines Prices Review Board (PMPRB) is to protect the interest of Canadian consumers by ensuring that the prices of patented medicines sold in Canada are not excessive. The affordability of medications is important; however, this cannot be considered in isolation of other factors, such as access. We are concerned that the Proposed Amendments will have a negative effect on the overall length of time for medications to reach Canadian patients.

Making a medication affordable does not improve health outcomes of Canadians if the drug ultimately does not launch in the Canadian market at all. New regulatory factors have the potential to significantly impact how Canada will be compared to the median of the newly proposed 12 OECD countries, the PMPRB12. On the surface, this may represent lower drug costs at the point of sale, however, we are gravely concerned about the unintended consequences that may not be visible immediately. How will these changes impact the length of time it takes for new medicines to be launched in the Canadian market, or whether or not, our country is selected for investment in research and clinical trials by international organizations?

A greater analysis of the real costs associated with these changes is highly advised. Key stakeholders who are impacted by these decisions need to be meaningfully engaged and included in a constructive dialogue to identify ways that Canada can strengthen our position as a leader in providing access to affordable medicines for Canadians. We strongly encourage the Federal Government to postpone updating the *Regulations* until such a time that the full impact of these changes are understood and solutions to these risks are identified.

3. Gaps in Pharmaco-economic Assessments for Patients

The introduction of new economics-based factors will see the PMPRB utilizing pharmaco-economic assessments that are used by the Canadian Agency for Drugs and Technologies in Health (CADTH) for the purposes of determining clinical and cost-effectiveness of a medication. This proposed method will be used as a factor for establishing a ceiling price for medications. This is of particular concern to the health charities and to many patient groups, as Quality Adjusted Life Year (QALY) assessments do

not include metrics that are important to patients, such as frequency of taking medications and quality-of-life measures.

QALY assessments do not have favourable outcomes for patients who require rare disease medicines, known as orphan therapies. Using this methodology, QALYs, orphan therapies are typically found to be "cost-ineffective" and lacking in long-term data on safety and effectiveness relative to other conditions and disease. This is indicative of a system limitation of the method, rather than the medicines. If the PMPRB relies on the same methods as CADTH, the ceiling price could be expected to be set at a level which could make access and availability even more difficult than it is currently. It is feared that implementing this change in the *Regulations* will only widen the gaps of equity and access to Canadians. We highly recommend that pharmaco-economic assessments not be included as a new regulatory factor.

4. Reaffirmed Commitment to Consultation

HCCC and its members were pleased to provide comment and input into the PMPRB consultation in June. Participation in the consultation was under the premise that all stakeholder feedback would be considered and valued. The PMPRB website indicates that the PMPRB is "committed to listening to the voices and views of Canadians, and to including them in decision making. Effective and meaningful stakeholder involvement is essential to enable the PMPRB to fulfil its mandate, deliver programs, launch new initiatives, and build public trust."[1] After providing a comprehensive list of recommendations in June, we were incredibly disappointed that our feedback was summarized into one paragraph in the Gazette and no notable changes were made to the Proposed Amendments based on the feedback that was provided.

We believe that this is not in keeping with the spirit that was intended, and ask that the Government of Canada commit to:

a) Establishing a formal mechanism that meaningfully and continuously engages patient representatives and other key stakeholders in the decision-making and regulatory processes, and

b) Ensure that future updates to the *Regulations* be undertaken in a fully transparent manner that clearly details the nature of the changes, provides all relevant information publicly, provides sufficient time for stakeholder input, dialogue, and exchange as part of the decision-making process.

We look forward to a continued dialogue on the affordability, availability and access to medicines for Canadians as one discussion cannot happen in isolation of the other. Thank you for your consideration of our feedback.

Sincerely,

Tammy Moore
CEO

[1] Retrieved (February 1 2018) from: http://www.pmprb-cepmb.gc.ca/news-and-events/consultations